(PB4267) BETIBEGLOGENE AUTOTEMCEL STEM CELL COLLECTION IN THREE Β-THALASSEMIA PATIENTS

Topic: 32. Transfusion medicine

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Background

Betibeglogene autotemcel (beti-cel) is an autologous gene-therapy product requiring robust mobilization and collection of CD34+ hematopoietic stem cells. Ensuring efficient apheresis yield is critical for successful product manufacturing.

Aims

We report three real-world cases of beti-cel collections performed in two patients with E-β thalassemia and one with β thalassemia major, highlighting mobilization approaches, collection characteristics, and CD34+ cell yields.

Methods

Data were reviewed from three individuals: a 6-year-old male with E-β thalassemia, a 28-year-old female with β-thalassemia major, and a 31-year-old female with E-β thalassemia. All underwent mobilization with daily G-CSF starting 4 days prior to first collection day through the last collection day. Plerixafor was given 4.5 to 6 hours prior to the start of each collection. Apheresis was performed on consecutive collection days using Spectra Optia (software version 12.1) and ACD-A anticoagulant ratio of 10:1.

Results

All three patients successfully completed mobilization and collection without reported adverse events. The 6-year-old male demonstrated the highest yield, with a mean total CD34+ collection of 54.4 $\times 10^6$/kg over three days, of which 45.6 $\times 10^6$/kg was sent (manufacturing incomplete at time of writing). The 28-year-old female collected 48.9 $\times 10^6$/kg over two days, with 35.3 $\times 10^6$/kg sent for manufacturing, and a manufactured beti-cel product of 7.85 $\times 10^6$/kg (22.2% of sent dose). One month post infusion this patient continues to be RBC transfusion dependent. The 31-year-old female achieved a total collection of 35.2 $\times 10^6$/kg over three days, with 30.0 $\times 10^6$/kg sent for manufacturing, and a manufactured beti-cel product of 7.85 $\times 10^6$/kg (26.1% of sent dose).

Summary/Conclusion

Across three diverse patients, mobilization using G-CSF with plerixafor produced good CD34+ yields for beti-cel manufacturing. These findings support the reliability of standard mobilization and apheresis protocols for robust gene-therapy collections in β-thalassemia major and E-β thalassemia patients.

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